

August 2014 Newsletter

CIRM August Newsletter

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CIRM-Funded Diabetes Therapy Takes Big Step Toward Human Trials



On July 17th, a promising treatment for type 1 diabetes that has received almost \$40 million in funding from CIRM took a major step towards being tested in people. ViaCyte Inc., the company behind the therapy, submitted an Investigational New Drug application (IND) with the Food and Drug Administration (FDA) seeking permission to start a phase 1/2 clinical trial in patients with type 1 diabetes (T1D). The trial is designed to see if their implantable product, the VC-01TM product candidate, is safe and shows evidence of effectiveness for patients with the disease. VC-01TM is composed of a thin plastic pouch, containing an immature form of pancreatic cells that, when implanted under the skin, are designed to mature and become insulin-producing cells. These cells are able to sense when blood glucose is high, and then secrete insulin to restore it to a healthy level. Clinical trials are expected to begin by the end of 2014.

- Read the our press release
- · Read our blog about this milestone
- Read more about VC-01[™] on Viacyte's website

Welcome to The Stem Cellar: CIRM's Revamped, Reimagined Blog

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We originally launched the CIRM blog as a way to share the latest and greatest news, events and insight related to the world of regenerative medicine. And while we've published more than 1,000 stories to date, and shared this knowledge with researchers, policymakers, advocates and—most importantly—patients, we wanted to take our blog to the next level. So, in July, after a lot of behind the scenes work, we rolled out The Stem Cellar, the official blog of California's Stem Cell Agency. Its new home is at blog.cirm.ca.gov.

- Read our blog about The Stem Cellar
- · Visit The Stem Cellar, CIRM's official blog

New CIRM President Takes Strong Stand on Ethics and Integrity



At the July 24th CIRM governing Board meeting, President C. Randal Mills, announced that he had instructed the agency's General Counsel to draw up an agreement indicating that he will not accept a job with any company funded by CIRM for at least one year following his departure, and will refuse to accept gifts or travel payments from any company, institution or individual who receives agency funding. The commitment from Mills takes effect immediately. "I want the people of California to know that my sole interest in being at CIRM is to help advance stem cell treatments to patients who are in need," said Dr. Mills. "I will do so with a full commitment to transparency and by never compromising the integrity of our mission nor our trust to the taxpayers of California."

· Read our press release and blog about Mills' commitment

Huntington's and Vision Loss Researchers Win Add-on Funds; Alpha Clinics Funding Modified

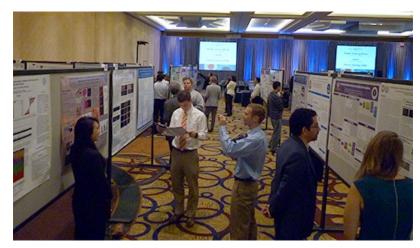
At the July 24th governing Board meeting, two CIRM-funded projects were awarded Bridging awards to help them carry out research that was not covered by earlier CIRM funding. Leslie Thompson, Ph.D., from the University of California, Irvine, was awarded more than \$500,000 to conduct laboratory tests of a potential therapy for Huntington's disease, a devastating and always fatal brain disorder. Currently there are no effective treatments for Huntington's. Sophie Deng, M.D., Ph.D., a researcher at the University of California, Los

Angeles, was awarded almost \$700,000 for work in developing a synthetic scaffold to be used in advancing our knowledge of Limbal Stem Cell Deficiency, a blinding eye disorder, generally caused by damage to the cornea on the surface of the eye.

In other actions the agency's Board approved President Mill's recommended change in the Alpha Stem Cell Clinic concept plan, agreeing to a modified version of the Coordinating and Information Management Center with a reduced budget of \$10 million. President Mills explained that the plan was worthy of funding but "the proposal as written is too broad and overly complex to be successful."

• Read our press release

CIRM Brings Together New Generation of Stem Cell Scientists for Annual Meeting



On July 28th and 29th, CIRM held its annual Bridges Trainee Meeting in Burlingame, CA. The conference brings together more than 150 of the Bridges to Stem Cell Research students along with some of the leading stem cell researchers from around California. It's a chance for these undergraduate and master's level students to present research results from their internships and speak with CIRM-funded scientists and CIRM science officers. CIRM has invested more than \$50 million in this six year program, so far helping more than 600 students to do great research at over 60 different academic institutions and biotech companies around California. Half of former Bridges trainees are employed in lab jobs and over 25% have enrolled in graduate or professional schools with 7% currently applying.

Read our blog about this year's Bridges meeting

The Latest Publications on CIRM-funded Research

CIRM grantees are making steady progress on understanding the fundamentals of stem cell biology and moving stem cell-based therapies toward clinical trials. Here are examples of CIRM-funded research that was published in July along with press releases from the grantees' home institutions and entries from the Stem Cellar, CIRM's official blog:

July 3rd - Cell Stem Cell

- Salk Institute Release: No extra mutations in modified stem cells, study finds
- CIRM Blog: Tinkering with stem cell genes safe

July 4th - Journal of Materials Chemistry

CIRM Blog: (UCSD) Making stem cells feel like they are growing in the right neighborhood may be key to success

July 15th - Circulation Research

CIRM Blog: (Stanford) Stem Cells become Tool to Screen for Drugs; Fight Dangerous Heart Infections

July 15th - Journal of Neuroscience

- Gladstone Release: Transplantation of healthy new brain cells reverses learning and memory loss in Alzheimer's disease model
- CIRM Blog: What was Old is New Again: Scientists Transplant Brain Cells into Aged Mice and Reverse Memory Loss

July 29th - Translational Psychiatry

- Western University of Health Science Release: WesternU professor develops blood test for Alzheimer's disease risk
- CIRM Bloq: Blood Test Reveals Alzheimer's Disease Risk, CIRM-Funded Study Finds

July 31st - Cell Death and Disease

- Sanford-Burnham Medical Research Institute: A new way to generate insulin-producing cells in type 1 diabetes
- CIRM Blog: Research points to another path toward giving diabetics the insulin-producing cells they need

Upcoming CIRM Events

Sep 10th: The CIRM governing Board will hold its next meeting at the Claremont Hotel in Berkeley, CA. The agenda and details will be posted to the **meetings page 10** days before the event.

CIRM Funding Opportunities

Current Applications

PA 14-04: CIRM Extraordinary Supplement Awards - Prior to submitting an application, a PI must submit a Letter of Intent (LOI) describing the proposed project. CIRM will evaluate the LOI -- refer to Section V.A. of the PA for evaluation criteria. This PA is targeted at only the most exceptional proposals: CIRM expects to support only one or two such projects each year.

RFA 13-04: External Innovation Pilot Program - Prior to submitting an application, a PI must submit a Letter of Intent (LOI) describing the proposed project. CIRM will evaluate the LOI -- refer to Section VIII.A. of the RFA for evaluation criteria. This RFA is targeted at only the most exceptional proposals: CIRM expects to support only one or two such projects each year.

PA 08-06: CIRM Conference Grants - Applications for conference grants will be accepted, reviewed and approved periodically

Get more info about CIRM RFAs Sign up to receive CIRM funding announcements

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